

## SEQUENCE LISTING

- <110> Mitchell, Lloyd G. Garcia-Blanco, Mariano A. Puttaraju, Madaiah Mansfield, Gary S.
- <120> METHODS AND COMPOSITIONS FOR USE IN SPLICEOSOME MEDIATED RNA TRANS-SPLICING

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<130> A31304-B-A-B 072874.0135
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<140> 09/756,096

<141> 2001-01-08

<150> 09/158,863

<151> 1998-09-23

<150> 09/133,717

<151> 1998-08-13

<150> 09/087,233

<151> 1998-05-28

<150> 08/766,354

<151> 1996-12-13

<150> 60/008,317

<151> 1995-12-15

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tccattcaaa aa

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<213> Corynebacterium diptheriae
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<210> 3
<211> 36
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ttcctgca
68
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tcgagaacat tattataacg ttgc
24
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18
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<211> 16
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<212> DNA
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<400> 23
gaaggctgag cactacacgc
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<212> DNA
<213> Homo sapien
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<212> DNA
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19
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<211> 39
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39
<210> 29
<211> 36
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<213> Artificial Sequence
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<223> Oligonucleotide primer complimentary to the
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<210> 30
<211> 38
<212> DNA
<213> Artificial Sequence
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<220>
<223> Oligonucleotide primer complimentary to the
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<211> 38
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<223> Oligonucleotide primer complimentary to the
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<400> 31
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38
<210> 32
<211> 47
<212> DNA
<213> Artificial Sequence
<220>
<223> Oligonucleotide primer complimentary to the
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47
<210> 33
<211> 37
<212> DNA
<213> Artificial Sequence
<220>
<223> Oligonucleotide primer complimentary to the beta
      HCG6 gene (accession #X00266)
<400> 33
gcatggatcc tccggagggc ccctgggcac cttccac
37
<210> 34
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<211> 38
<212> DNA
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<220>
<223> Oligonucleotide primer complimentary to the beta
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<400> 34
ctgactgcag ggtaaccgga caaggacact gcttcacc
<210> 35
<211> 35
<212> DNA
<213> Artificial Sequence
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<223> Oligonucleotide primer complimentary to the beta
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<400> 35
gcatggtaac cctgcagggg ctgctgctgt tgctg
<210> 36
<211> 37
<212> DNA
<213> Artificial Sequence
<220>
<223> Oligonucleotide primer complimentary to the beta
      HCG6 gene (accession #X00266)
<400> 36
ctgaaagctt gttaaccagc tcaccatggt ggggcag
<210> 37
<211> 22
<212> DNA
<213> Artificial Sequence
<220>
<223> Oligonucleotide primer complimentary to the
      Escherichia coli lacZ gene
<400> 37
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<210> 38
<211> 21
<212> DNA
<213> Artificial Sequence
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<223> Oligonucleotide primer complimentary to the
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<400> 38
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21
<210> 39
<211> 20
<212> DNA
<213> Artificial Sequence
<220>
<223> Oligonucleotide primer complimentary to the
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<400> 39
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<212> DNA
<213> Homo sapien
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<211> 35
<212> DNA
<213> Homo sapiens
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<210> 42
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<211> 30
<212> DNA
<213> Homo sapiens
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<210> 43
<211> 51
<212> DNA
<213> Homo sapien
<400> 43
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51
<210> 44
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<213> Homo sapien
<400> 44
gacctctcga gggatttggg gaattatttg ag
32
<210> 45
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35
<210> 46
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<400> 48
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21
<210> 49
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<400> 49
cgcctaatga tgatgatgat g
21
<210> 50
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<213> Homo sapien
<400> 50
cttcttggta ctcctgtcct g
21
<210> 51
<211> 32
<212> DNA
<213> Homo sapien
<400> 51
gacctctcga gggatttggg gaattatttg ag
32
<210> 52
<211> 21
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<213> Homo sapien
<400> 52
aactagaagg cacagtcgag g
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21
<210> 53
<211> 24
<212> DNA
<213> Artificial Sequence
<220>
<223> trans-spliced product containing Human chorionic
      gonadotropin gene 6 sequences and Corynebacterium
      diptheriae diptheria toxin A sequence
<400> 53
gagatgttcc agggcgtgat gatg
24
<210> 54
<211> 127
<212> RNA
<213> Artificial Sequence
<220> .
<223> PTM intramolecular base paired stem
<221> misc_feature
<222> (57)...(70)
<223> Loop comprising a combination of 14 nucleotides
      according to specification
<400> 54
gcuagecugg gacaaggaca cugcuucace egguuaguag accaeagece ugageennnn
nnnnnnnnn aucguuaacu aauaaacuac uaacugggug aacuucuguu uuuuucucga
120
gcugcag
127
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<211> 127
<212> RNA
<213> Artificial Sequence
<220>
<223> PTM intramolecular base paired stem
<221> misc feature
<222> (57)...(70)
<223> Loop comprising a combination of 14 nucleotides
```

## according to specification

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<400> 55
gcuagecugg gacaaggaca cugcuucacc cgguuaguag accacagccc uqaqccnnnn
nnnnnnnnn aucguuaacu aauaaacuac uaacugggug aacuucugua uuauucucga
120
gcugcag
127
<210> 56
<211> 127
<212> RNA
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<223> PTM intramolecular base paired stem
<221> misc feature
<222> (57)...(70)
<223> Loop comprising a combination of 14 nucleotides
      according to specification
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nnnnnnnnn aucguuaacu aauaaacuac uaacugggug aaguucuguc cuugucucga
120
gcugcag
127
<210> 57
<211> 132
<212> DNA
<213> Artificial Sequence
<220>
<223> trans-spliced product containing Human chorionic
      gonadotropin gene 6 sequences and Corynebacterium
      diptheriae diptheria toxin A sequences
<400> 57
caggggacgc accaaggatg gagatgttcc agggcgctga tgatgttgtt gattcttctt
aaatcttttg tgatggaaaa cttttcttcg taccacggga ctaaacctgg ttatgtagat
120
tccattcaaa aa
132
```

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<400> 58
gaattcggta ccatgggg
18
<210> 59
<211> 33
<212> DNA
<213> Artificial Sequence
<220>
<223> Artificial Sequence derived from Escherichia coli
      lacZ gene
<400> 59
cgtttacagg taagaggatc ctccggaggg ccc
<210> 60
<211> 30
<212> DNA
<213> Artificial Sequence
<220>
<223> Artificial Sequence derived from Escherichia coli
      lacZ gene
<400> 60
tggtgtcaaa aataataagt taacaagctt
30
<210> 61
<211> 25
<212> DNA
<213> Artificial Sequence
<220>
<223> trans-spliced product containing Escherichia coli
      lacZ gene sequences and Human chorionic
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## gonadotropin gene 6 exon 2 sequences <400> 61 cagcagcccc tgtaaacggg gatac 25 <210> 62 <211> 286 <212> DNA <213> Artificial Sequence <220> <223> trans-spliced product containing Escherichia coli lacZ gene sequences <400> 62 ggctttcgct acctggagag acgcgcccgc tgatcctttg cgaatacgcc cacgcgatgg gtaacagtct tggcggtttc gctaaatact ggcaggcgtt tcgtcagtat ccccgtttac 120 agggcggctt cgtctaataa tgggactggg tggatcagtc gctgattaaa tatgatgaaa acgggcaacc cgtggtcggc ttacggcggt gattttggcg atacgccgaa cgatcgccag 240 ttctgtatga acggtctggt ctttgccgac cgcacgccgc atccag <210> 63 <211> 196 <212> DNA <213> Artificial Sequence <220> <223> trans-spliced product containing Escherichia coli lacZ gene sequences <400> 63 ggettteget acetggagag acgegeeege tgateetttg egaataegee eacgegatgg gtaacagtet tggeggttte getaaataet ggeaggegtt tegteagtat eecegtttae aggggctgct gctgttgctg ctgctgagca tgggcgggac atgggcatcc aaggagccac ttcggccacg gtgccg 196 <210> 64

<211> 420

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<213> Artificial Sequence
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<223> trans-spliced product comprising cystic fibrosis
      transmembrane regulator-derived sequences and His
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aacgttgctc gagtactaac tggaacctct tcttttttt cctgcagact tcacttctaa
120
tgatgattat gggagaactg gagccttcag agggtaaaat taagcacagt ggaagaattt
cattetgtte teagttttee tggattatge etggeaceat taaagaaaat ateatetttg
gcggccgcca ctgtgctgga tatctgcaga attccaccac actggactag tggatccgag
300
ctcggtacca aggttaagtt taaaccgctg atcagcctcg actgtgcctt ctagttgcca
gccatctgtt gtttgcccct cccccgtgcc ttccttgacc ctggaaggtg ccactcccac
420
<210> 65
<211> 20
<212> DNA
<213> Artificial Sequence
<220>
<223> Splice junction sequence
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20
<210> 66
<211> 7
<212> PRT
<213> Artificial Sequence
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<223> C terminal residues from glutathione -S-
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<400> 66
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.

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1
<210> 67
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<212> DNA
<213> Artificial Sequence
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<223> Artificial sequence comprising sequences derived
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<400> 67
ggagttgatc ccgtc
15
<210> 68
<211> 37
<212> DNA
<213> Artificial Sequence
<220>
<223> Artificial sequence comprising sequences derived
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<212> DNA
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gattcacttg ctccaattat catcctaagc agaagtgtat attcttattt gtaaagattc
tattaactca tttgattcaa aatatttaaa atacttcctg tttcatactc tgctatgcac
<210> 70
<211> 24
<212> DNA
<213> Artificial Sequence
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24
<210> 71
<211> 47
<212> DNA
<213> Artificial Sequence
<223> Branch point, pyrimidine tract and acceptor splice
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tactaactgg tacctcttct tttttttttg atatcctgca gggcggc
47
<210> 72
<211> 70
<212> DNA
<213> Artificial Sequence
<223> Donor site and spacer sequence of PTM
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tgaacggtaa gtgttatcac cgatatgtgt ctaacctgat tcgggccttc gatacgctaa
60
gatccaccgg
70
<210> 73
<211> 260
<212> DNA
<213> Artificial Sequence
<223> Binding domain of spacer sequence
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gtatctatat tcatcattgg aaacaccaat gatttttctt taatggtgcc tggcataatc
120
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ctggaaaact gataacacaa tgaaattctt ccactgtgct taaaaaaacc ctcttgaatt
ctccatttct cccataatca tcattacaac tgaactctgg aaataaaacc catcattatt
aactcattat caaatcacgc
260
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<223> Oligonucleotide primer
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cgctggaaaa acgagcttgt tg
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<210> 75
<211> 23
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actcagtgtg attccacctt ctc
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<223> Oligonucleotide
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<210> 77
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ctaggatccc gttcttttgt tcttcactat taa
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<210> 78
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ctagggttac cgaagtaaaa ccatacttat tag
33
<210> 79
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<210> 81
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<212> DNA
<213> Artificial Sequence
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<223> Binding domain of PTM molecule
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<210> 82
<211> 22
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gatcaaatct gtcgatcctt cc
22
<210> 83
<211> 21
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<213> Artificial Sequence
<220>
<223> Oligonucleotide primer
<400> 83
ctgatccacc cagtcccatt a
<210> 84
<211> 22
<212> DNA
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<220>
<223> Oligonucleotide primer
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gactgatcca cccagtccca ga
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<210> 85
<211> 52
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<220>
<223> Random sequence inserted to replace 3' splice site
<221> misc feature
<222> (7)...(30)
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ccgcggnnnn nnnnnnnnn nnnnnnnnn gggttccggt accggcggct tc
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tatgatgaaa a
71
<210> 87
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acgccg
66
<210> 88
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<220>
<223> PTM sequences
<400> 88
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aacataatct tcggcgtcag ttacgacgag taccgctatc gctcggtgat taaggcctgt
180
cagttggagg ag
192
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gagcaggcaa gacgagcttg ctcat
25
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<223> Oligonucleotide
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gagaacataa tcttcggcgt cagttacg
28
<210> 91
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<223> Oligonucleotide
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gtcagttgga ggaggacatc tccaagtttg
30
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120
aacataatct tcggcgtcag ttacgacgag taccgctatc gctcggtgat taaggcctgt
180
cagttggagg ag
192
<210> 93
<211> 27
<212> DNA
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<223> PTM sequences
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aaatatcatt ggtgtttctt atgatga
27
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<223> Oligonucleotide
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ccaactagaa gaggacatct ccaagtttgc
<210> 95
<211> 30
<212> DNA
<213> Artificial Sequence
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<223> Oligonucleotide
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atgatcatgg gcgagttaga accaagtgag
30
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<223> Oligonucleotide
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27
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<223> Oligonucleotide
<400> 97
ccaactagaa gaggacatct ccaagtt
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<212> DNA
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<223> 5' splice site
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cgtttacagg taagtggatc c
21
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<223> 3' splice site
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ctgcagggcg gcttcgtcta ataatgg
27
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<211> 1584
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<213> Artificial Sequence
<220>
<223> CFTR PTM
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120
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480
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840
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1440
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1584
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<211> 323
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323
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165
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aaatacttcc tgtttcacct actctgctat gcacccgcgg aacattatta taacgttgct
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225
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120					
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180		annatttaan	~~~~~~~	atatagttgt	tagagaagat
caactagaag 240	aggacatete	caagtttgca	gagaaagaca	atatagttct	cggagaagge
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